

# **Overview: motor assessment and rehabilitation**

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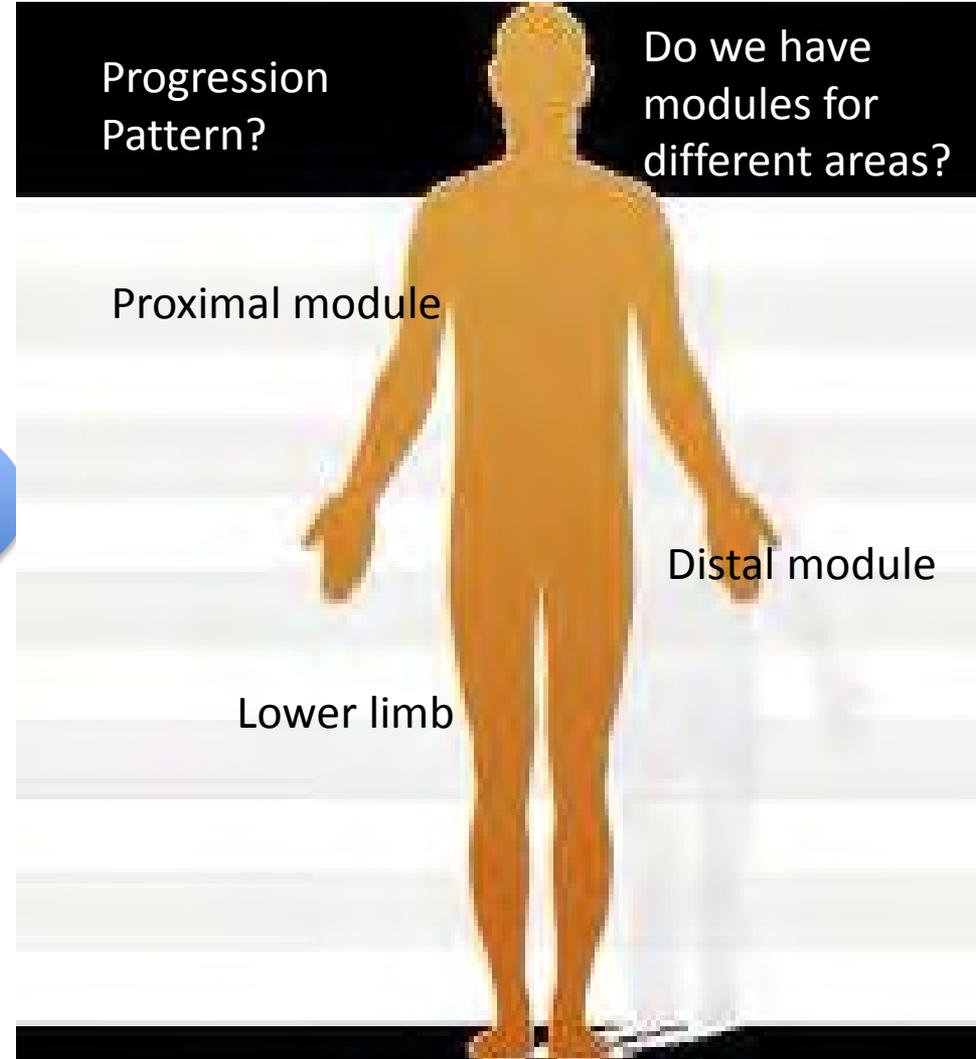
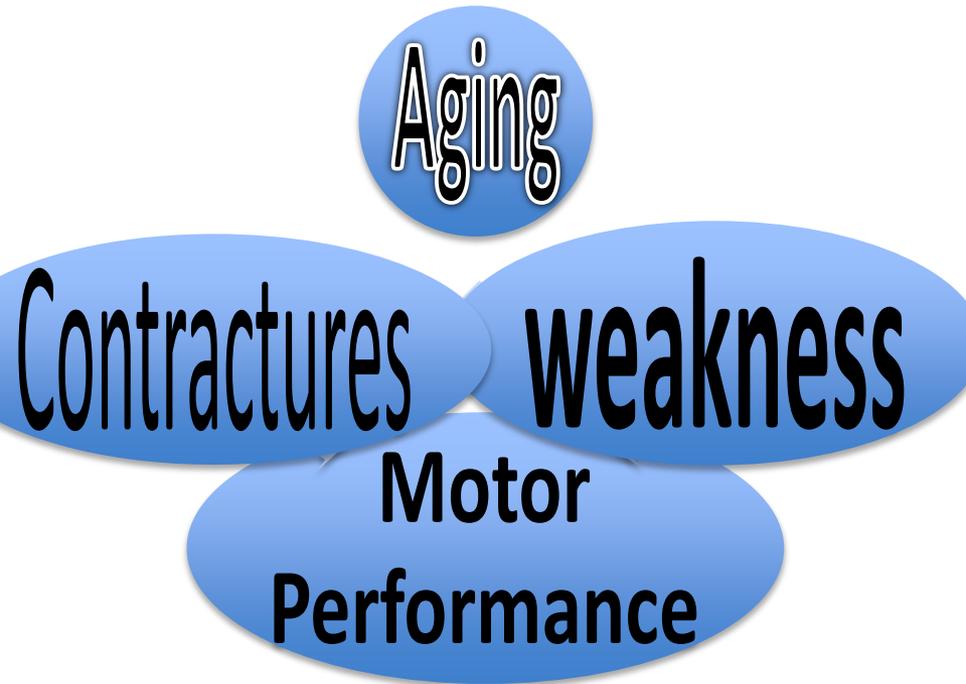
# Assessments for a clinical trial in calpainopathy

- We don't have strong Natural history data from which we can design outcome measures for a clinical trial
- We need to start gathering data internationally
- One of the first steps would be to gather patients representatives, expert physiotherapists and research clinicians with clinical trial experience to evaluate current generic and other disease specific outcome measures that may be appropriate in calpainopathy

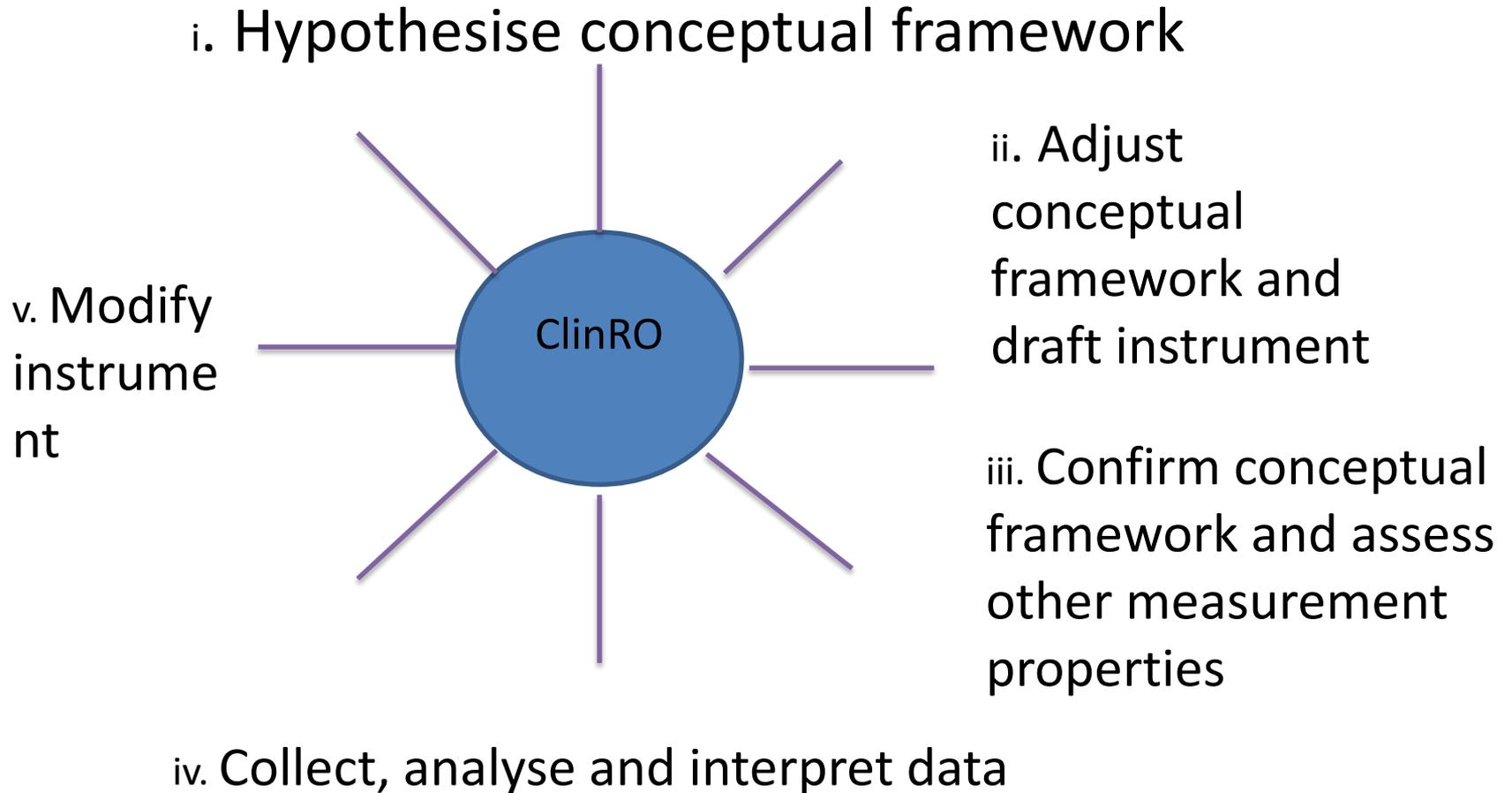
# Learn from other diseases

- In DMD and SMA trials were delayed because we did not have specific outcome measures and insufficient natural history data
- A trial can fail not because the drug doesn't work but because the outcome measures are inappropriate or not sensitive enough
- In dysferlinopathy, LGMD21, DMD, SMA there are projects specifically designed to develop trial ready assessments for their conditions

# Calpainopathy Conceptual Framework



# ClinRO – A road map for standardisation?



# Moving forward – what has been successful in other diseases?

## **Clinical Outcomes Study in Dysferlinopathy**

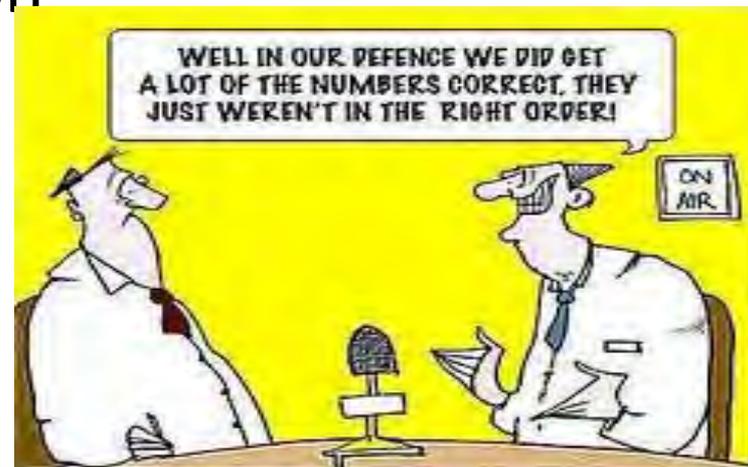
- Clinical expert group met in 2010 to deliberate which assessments were appropriate
- The situation was exactly as we have for calpainopathy now
- Little evidence for discriminatory tests in dysferlinopathy
- The functional measures that were chosen were in order to evaluate the dysferlin study population in the absence of any suitable disease specific assessments.

# Dysferlinopathy COS

- Muscle strength tests were based on the pattern of weakness
- The aim was to collect data for the first year of the study and then to objectively evaluate the efficacy of the assessments
  - Robustness -Relevance -Feasibility - ability to detect change
- Then to *make decisions* about which assessments to continue with and whether other assessments needed to be added in

# Role of the Physiotherapist in Clinical trials

- The clinical evaluator is a key person
- They gather the data that is used to determine whether a trial is a success or fails
- Physiotherapists have developed many of the OM used in current trials
- and standardise their application



# Rasch Analysis of MFM and NSAA in the COS study (one year data)

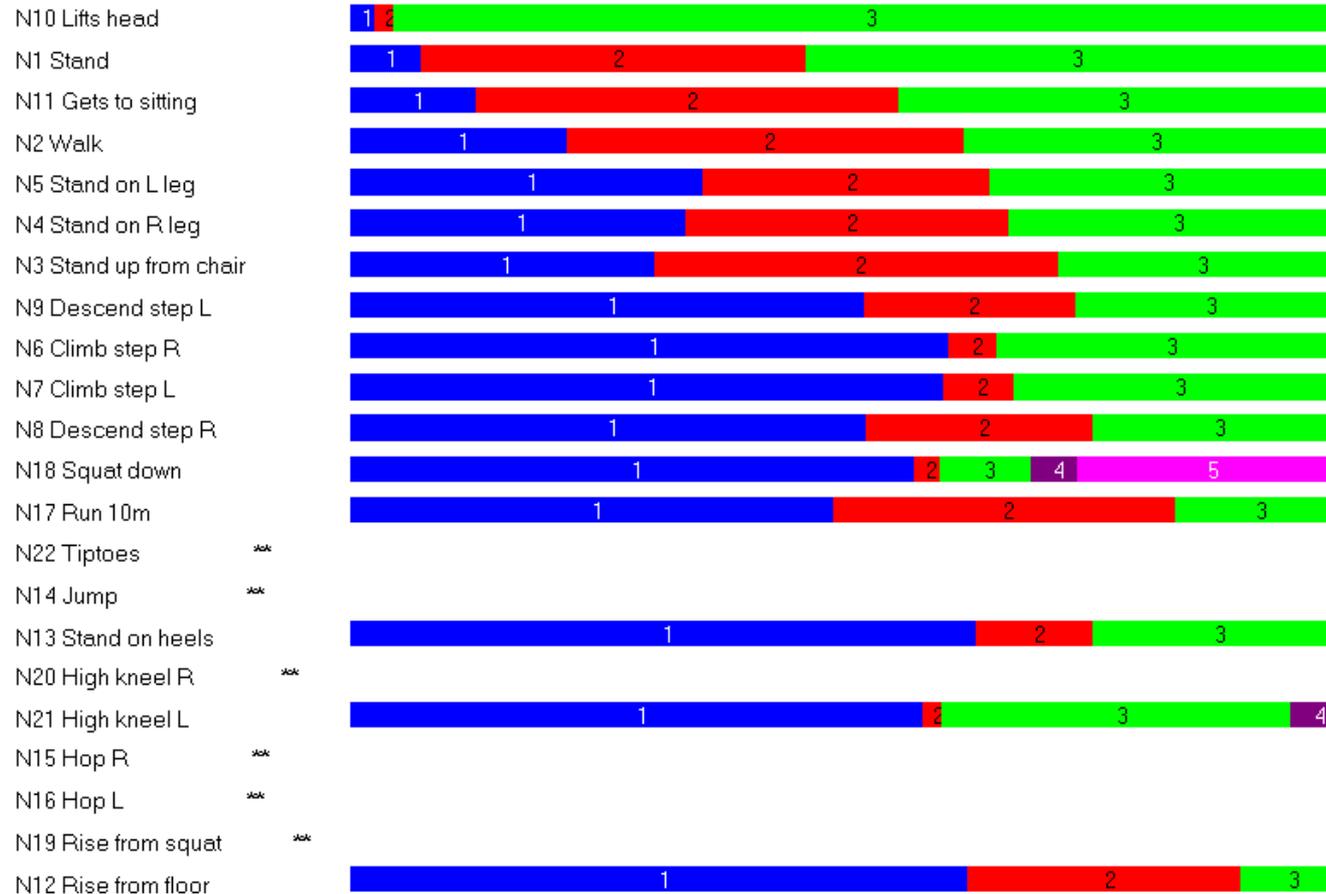
This analysis investigated the ability of the individual items of both scales to measure the underlying construct of the scale,

1. Whether the items target the range of abilities of the individuals
2. Whether the items order of difficulty match clinical knowledge of the condition.
3. It also reviewed the possibility of combining the scales to create a robust scale suitable for all levels of ability.

*Analysis was performed by Anna Mayhew and Meredith James  
Physiotherapists at the John Walton Newuromsucle Centre at  
Newcastle*

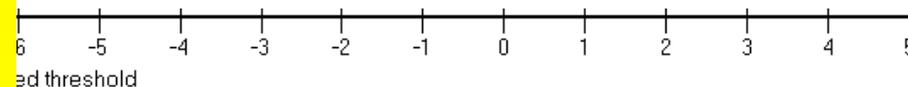
# NSAA for dysferlin Ambulant patients

## Threshold map



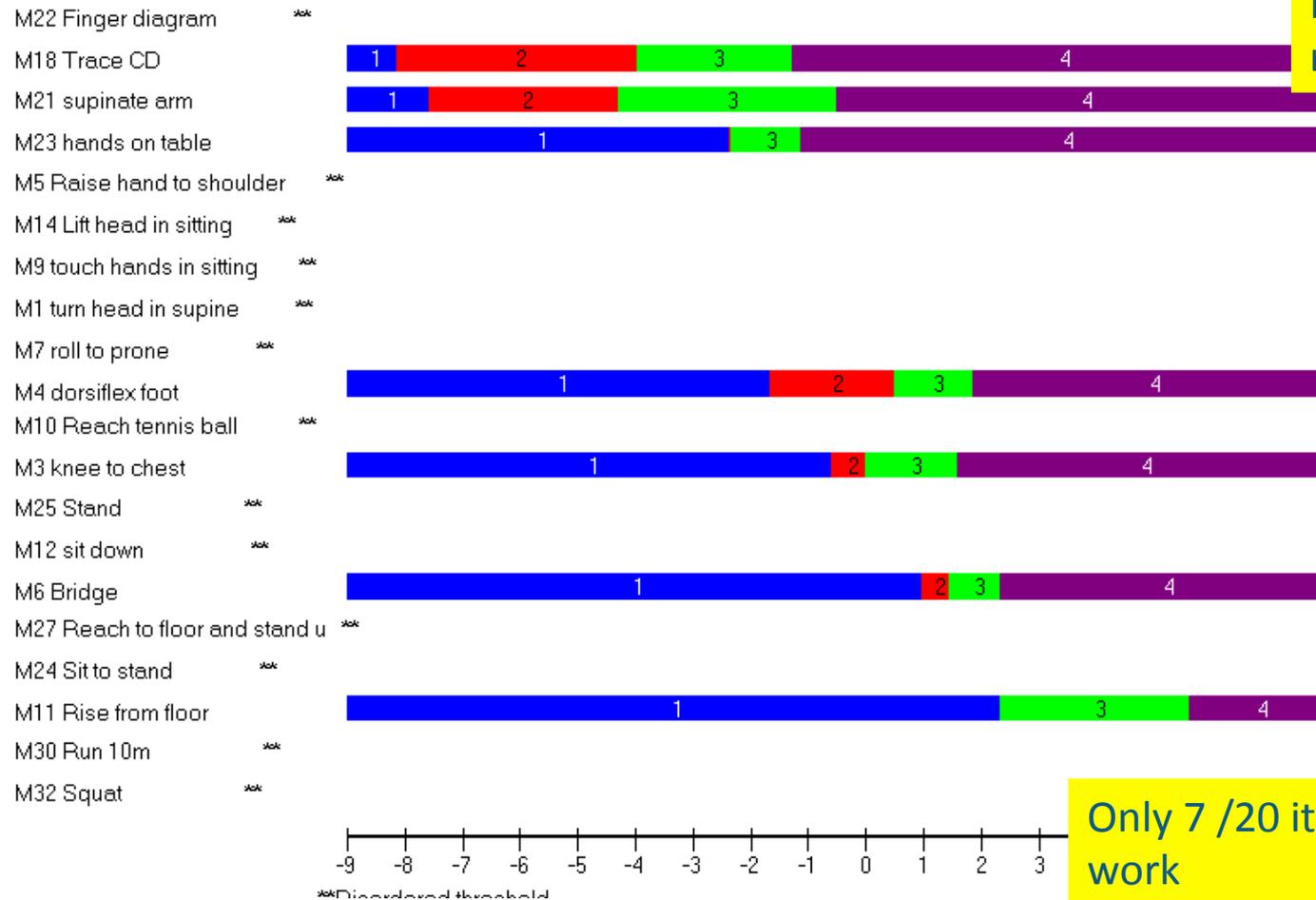
Item fit was very good

Original NSAA items work well. Scoring too complex for additional items



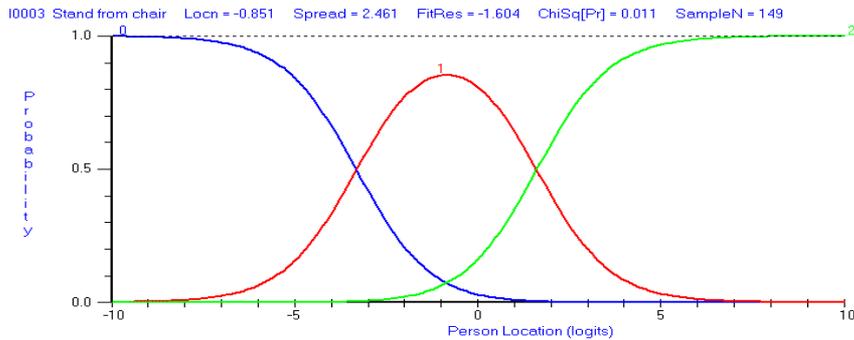
# MFM for all patients

## Threshold map

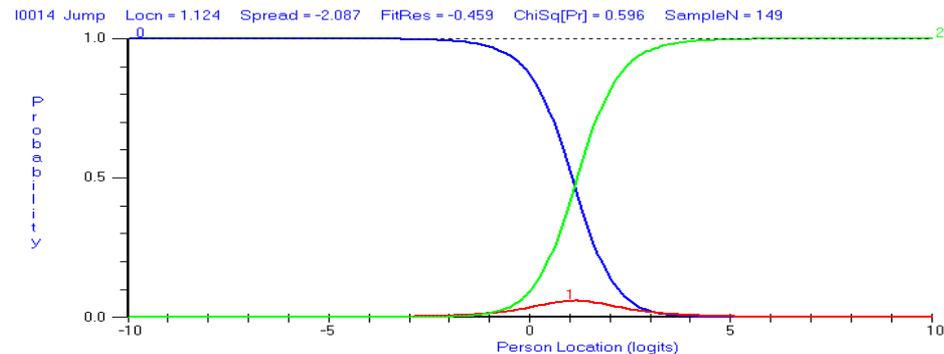


Only 7 /20 item thresholds work  
Scoring doesn't work well for this disease

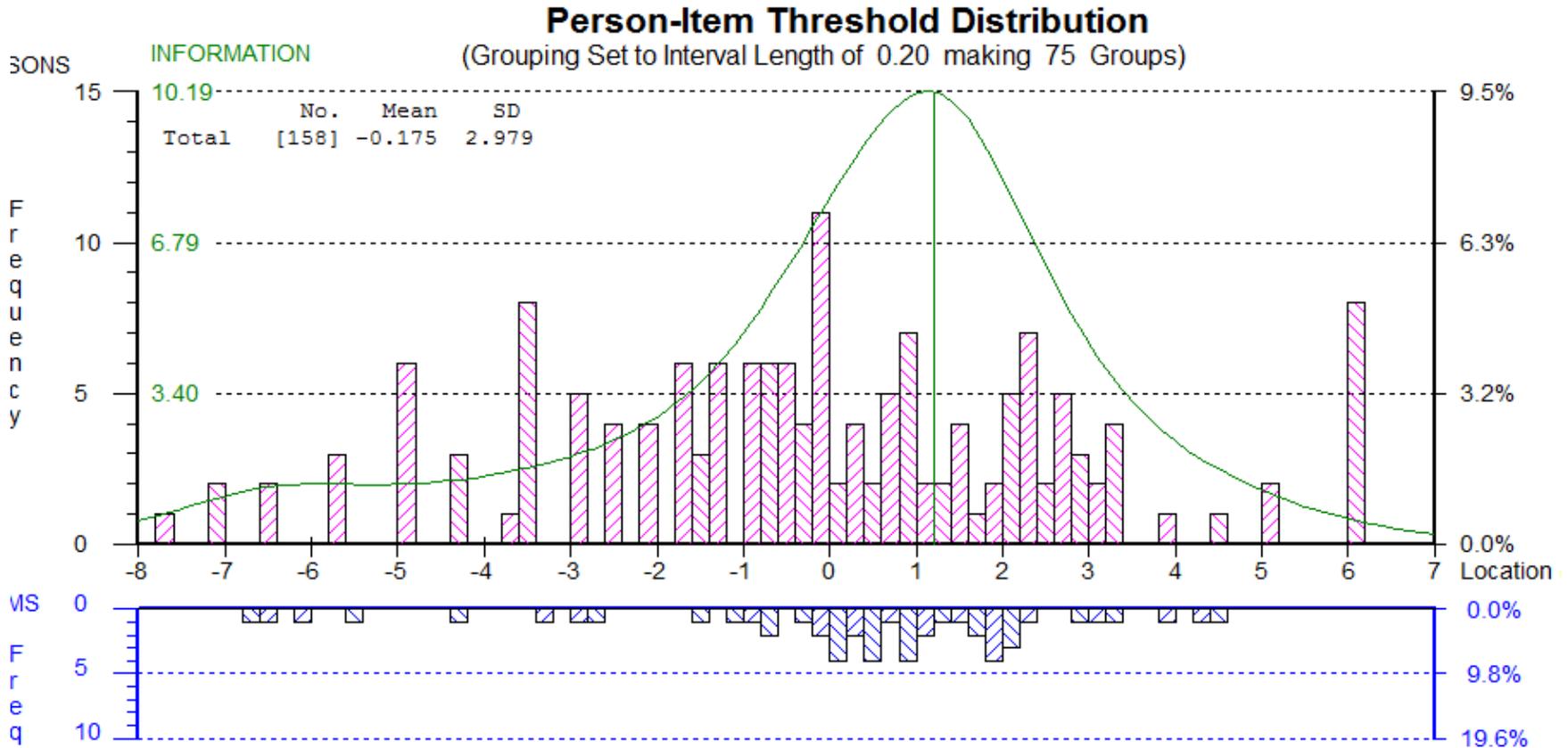
**Figure 1. Category Probability Curve for Stand up from chair showing logical progressive order.**



**Figure 2: Category Probability Curve disordered for jumping suggesting you can either do it (score 2) or not (score 0) but not with a skip (score 1)**

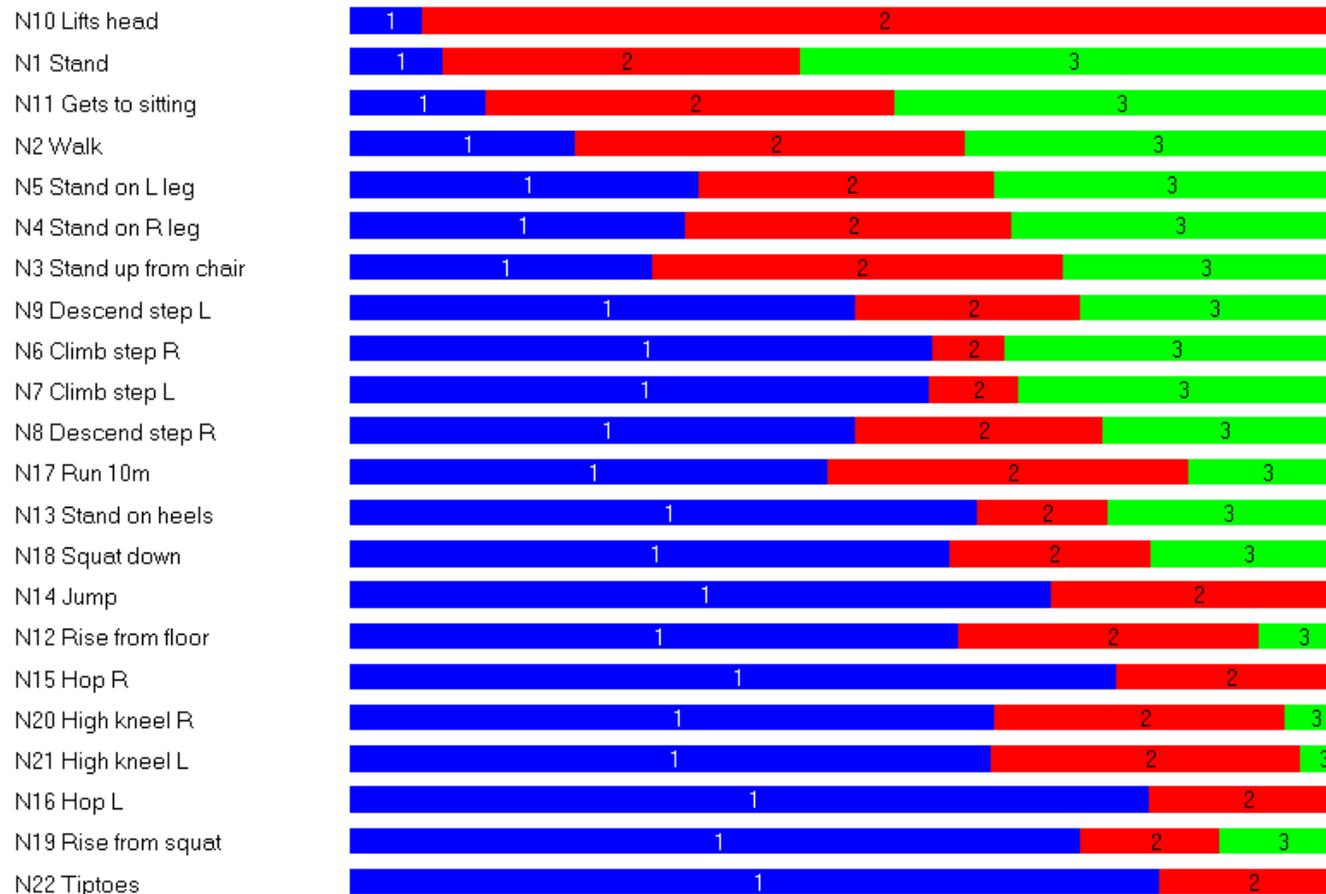


# Targeting of NSAA



Reasonable spread of items but a ceiling effect exists

# Re-score and addition of some MFM items of NSAA to become North Star for Dysferlinopathy



# Possible outcome measures in Calpainopathy

## ClinRo (clinician reported OMs)

## Patient reported outcome measures

### Strength

- manual muscle testing
- Hand held dynamometry
- Quantitative muscle strength testing

### Function

- A Calpainopathy version of NSAA
- Motor function measure
- Gross motor function measure
- Performance of the Upper Limb
- Active seated functional reach

### Timed tests

- Rise from floor
- Timed up and go
- 10 metre run/walk
- Sit to stand

6 Minute walk test

Respiratory function?

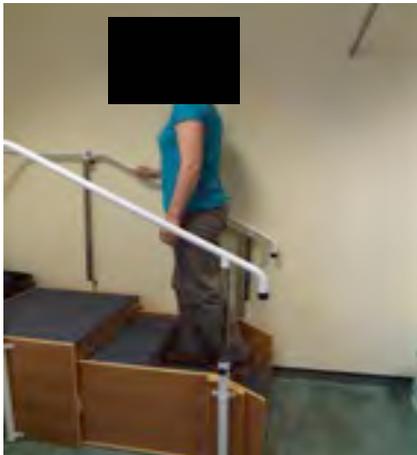


- Quality of life
- Function questionnaires e.g. Activlim
- PROM for Upper limb eg as recently designed for DMD
- Activity monitors?



# Functional Assessments

- Performance of the Upper Limb
- Extended North Star for Calpainopathy
- Timed stairs, TUG
- 6MWT



- ***To be relevant and meaningful for the patient the assessment must reflect the patient's function in the perspective of daily living (Fowler 1982)***

# Rehabilitation for Calpainopathy

- Currently physiotherapy is the only treatment for many of the neuromuscular diseases
- For any condition we should know the disease and what the likely complications are
- We can then plan a physiotherapy programme that is appropriate and that is forward looking
- It is easier to prevent a problem than it is to resolve it

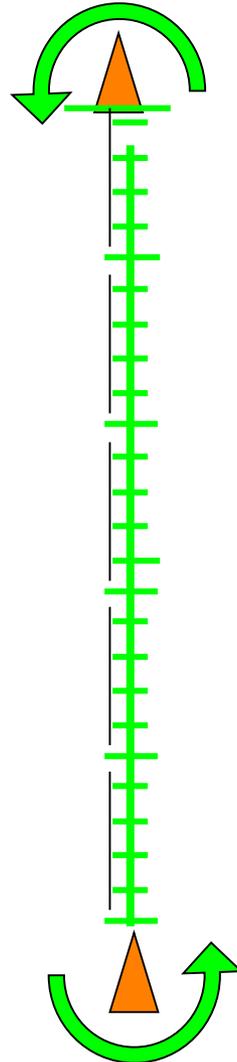
# What is physiotherapy? *Prevent, Maintain ?improve*

- Enabling a person through physical means to reach their potential and maintain function and ability
- **Anticipate** problems and be **pro-active** in managing them
- Assessment
- Exercise/rehabilitation
  - Stretching
  - Exercises/hydro/sports
  - Postural advice/wheelchairs and seating
  - Chest physiotherapy



# Assessment

- Functional Scales – North Star, PUL
- EK Scale
- timed tests – 10 metre, rise from floor, 6 MWT
- muscle strength - MMT, myometry, QMT
- Range of movement
- Respiratory - FVC, MIP/MEP, PCF



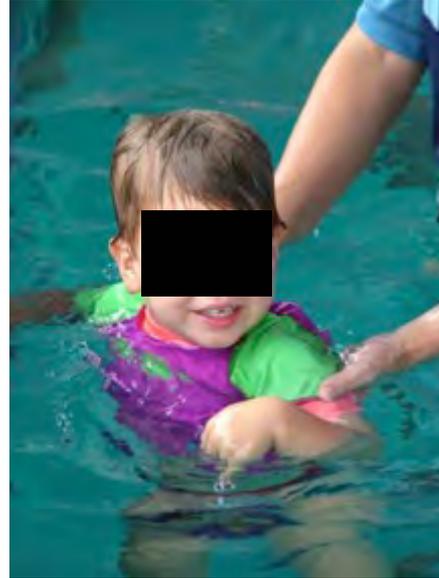
# Problems

- Loss of function
- Weakness
- Contractures
- Immobility



# Hydrotherapy

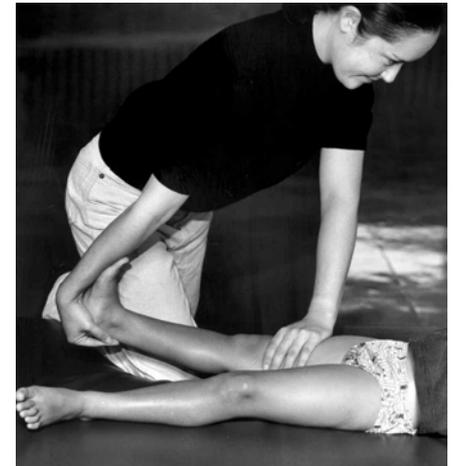
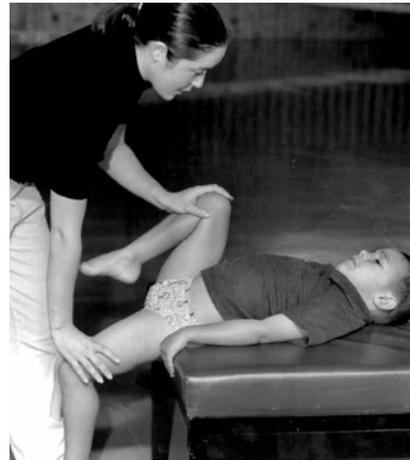
- Warm water is essential
- Most beneficial at any stage - ambulant or not
- Enables active concentric exercise and assists those muscles which are not able to counteract gravity on land
- Very little objective evidence in literature



# Why is stretching so important?

Contractures - an integral part of the disease in calpainopathy  
Elbows - hands - spine - knees - hips - ankles

- Prevents/delays permanent contractures
- Maintains comfortable movement
- Enables continued walking/standing
- More comfortable sleep
- Reduces asymmetry
- Enables nice shoes to be worn!



# Active exercise

- Healthy persons are advised to exercise aerobically 3 times a week for 30 minutes
- The same advice is given to people with a NMD
- Static cycle, swimming, cycling, walking



# Contracture correction devices

- Very little evidence on their use in NMD or DMD
- However there is some evidence that suggests that two hours per day (2x1 hour or 1x2 hours) is effective at improving range
- We have found these to be much more accepted and they have prevented deterioration and sometimes even improved ROM



# Standing/adjustable height wheelchairs

- Excellent
- Expensive
- Worth it
- Delay contractures
- Good for the spine
- Social/emotional benefits



# Powered Mobility



**People should have appropriate powered mobility once walking ability impacts on life style and quality of life**

# Sport and fun



England Wheelchair football team



# Work to be done

- We can be pragmatic from a clinical perspective
- For measurement – accuracy is key therefore knowledge of disease progression is key
- Only when we have robust, reliable repeatable standardised assessments can we evaluate the impact of any intervention be it pharmacological or physiotherapy

